

PCTWORLD INTELLECTUAL PROPERTY ORGANIZATION
International Bureau

INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(51) International Patent Classification ⁷ : C07K 16/00	A2	(11) International Publication Number: WO 00/24780 (43) International Publication Date: 4 May 2000 (04.05.00)
--	-----------	---

(21) International Application Number: PCT/US99/25091

(22) International Filing Date: 25 October 1999 (25.10.99)

(30) Priority Data:

60/105,731	26 October 1998 (26.10.98)	US
60/105,876	27 October 1998 (27.10.98)	US
60/141,175	25 June 1999 (25.06.99)	US

(71) Applicant (for all designated States except US): GENZYME CORPORATION [US/US]; Metrowest Place, 15 Pleasant Street Connector, Framingham, MA 01701-9322 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): IBRAGHI-MOV-BESKROVNAYA, Oxana [US/US]; Framingham, MA 01701 (US). VAN-DELLEN, K. [US/US]; Framingham, MA 01701 (US). PETRY, Linda, R. [US/US]; Framingham, MA 01701 (US).

(74) Agents: KONSKI, Antoinette, F. et al.; Baker & McKenzie, 660 Hansen Way, Palo Alto, CA 94304 (US).

(81) Designated States: AE, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CU, CZ, DE, DK, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, UA, UG, US, UZ, VN, YU, ZA, ZW, ARIPO patent (GH, GM, KE, LS, MW, SD, SL, SZ, TZ, UG, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG).

Published*Without international search report and to be republished upon receipt of that report.*

(54) Title: COMPOSITIONS AND METHODS FOR TREATING POLYCYSTIC KIDNEY DISEASE

(57) Abstract

The present invention provides an isolated antibody or fragment thereof that binds to an epitope present in the transmembrane domain of polycystin and specifically recognizes a polycystin-related polypeptide having an apparent molecular weight in the range of about 600 to about 800 kD. Polynucleotides, polypeptides, gene delivery vehicles and host cells containing the transmembrane sequences are also provided. Further provided are methods and compositions for modulating the biological activity of polycystin in a suitable cell or tissue.